

# Haemophilia Treatment A Global Perspective

*Brian O'Mahony, WFH President*

*HHS ACBSA January, 2004*





# PREVALENCE OF HEMOPHILIA

- Hemophilia A 105/Million males
- Hemophilia B 28/Million males
  
- Haemophilia A and B:
  - 399,000 people
  - 200,000 severe
  
- 105,000 known to WFH
  
- Von Willebrands: 600,000 people  
: 38,000 known



# Global Reality

- 75% not Diagnosed
- Many die in Childhood
- Hemophilia NOT a priority with Government
  - rare, expensive
- Lack of infrastructure, training, Education
- Cost of treatment prohibitive for individuals
- A safe supply of affordable replacement therapy currently reality only in developed countries



## Developed Countries

<u>Country</u>	<u>Population (millions)</u>	<u>% Diagnosed</u>	<u>No. HTC</u>	<u>Per Capita FVIII iu</u>
Australia	19	95	15	3.0
USA	278	87	140	3.4
Germany	82	82	6	5.5

*WFH Global Survey, 2002*



## Emerging Countries

<u>Country</u>	<u>Population (millions)</u>	<u>% Diagnosed</u>	<u>No. HTC</u>	<u>Per Capita FVIII iu</u>
Iran	63	82	10	0.5
Russia	146	81	4	0.1
Egypt	63	75	7	0.1
South Africa	42	52	10	0.6

*WFH Global Survey, 2002*



# WFH GLOBAL DATA 2001/2002

## Developing Countries

<u>Country</u>	<u>Population (millions)</u>	<u>% Diagnosed</u>	<u>No. HTC</u>	<u>Per Capita FVIII iu</u>
India	998	12	56	0.01
China	1227	5		
Indonesia	207	4	8	0.01
Bangladesh	128	2		0.002

*WFH Global Survey, 2002*



# COST OF REPLACEMENT THERAPY

- **Developed countries (WFH data):**

UK 120,000 I.U

Average cost US\$0.83 I.U.

Cost per person US\$100,000 p.a.

Country	GNP\$	Income Multiple
Australia	20,530	5
USA	29,080	3.3



# COST OF REPLACEMENT THERAPY

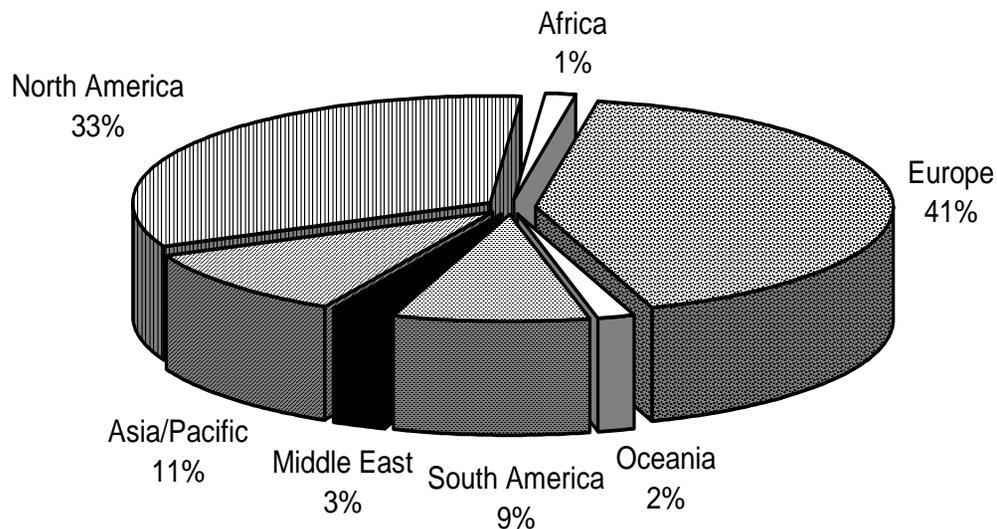
- **Developing Countries**
- Minimum on-demand 30,000 I.U.  
Cost US\$0.25 I.U.  
Cost per person US\$7,500 p.

<b>Country</b>	<b>GNP\$</b>	<b>Income Multiple</b>
India	370	20
Bangladesh	360	20
Indonesia	570	13
China	860	9



# Global use of Factor Concentrate

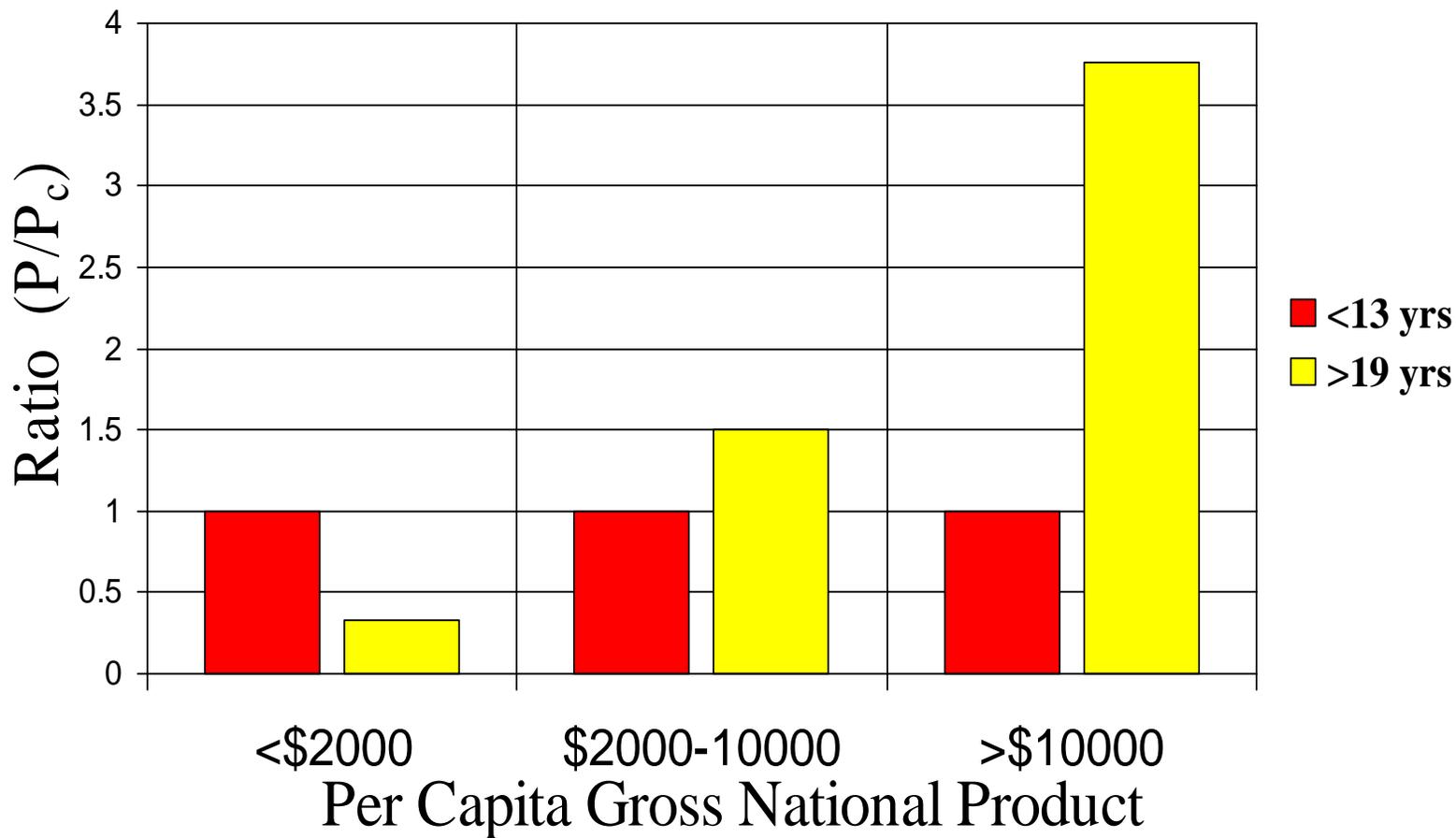
## REGIONAL DISTRIBUTION OF FACTOR VIII PLASMA-DERIVED AND RECOMBINANT (UNITS)



Total 2000: 3.7 billion international Units



# RELATIONSHIP OF ECONOMIC CAPACITY AND NUMBER OF ADULT HEMOPHILIA PATIENTS





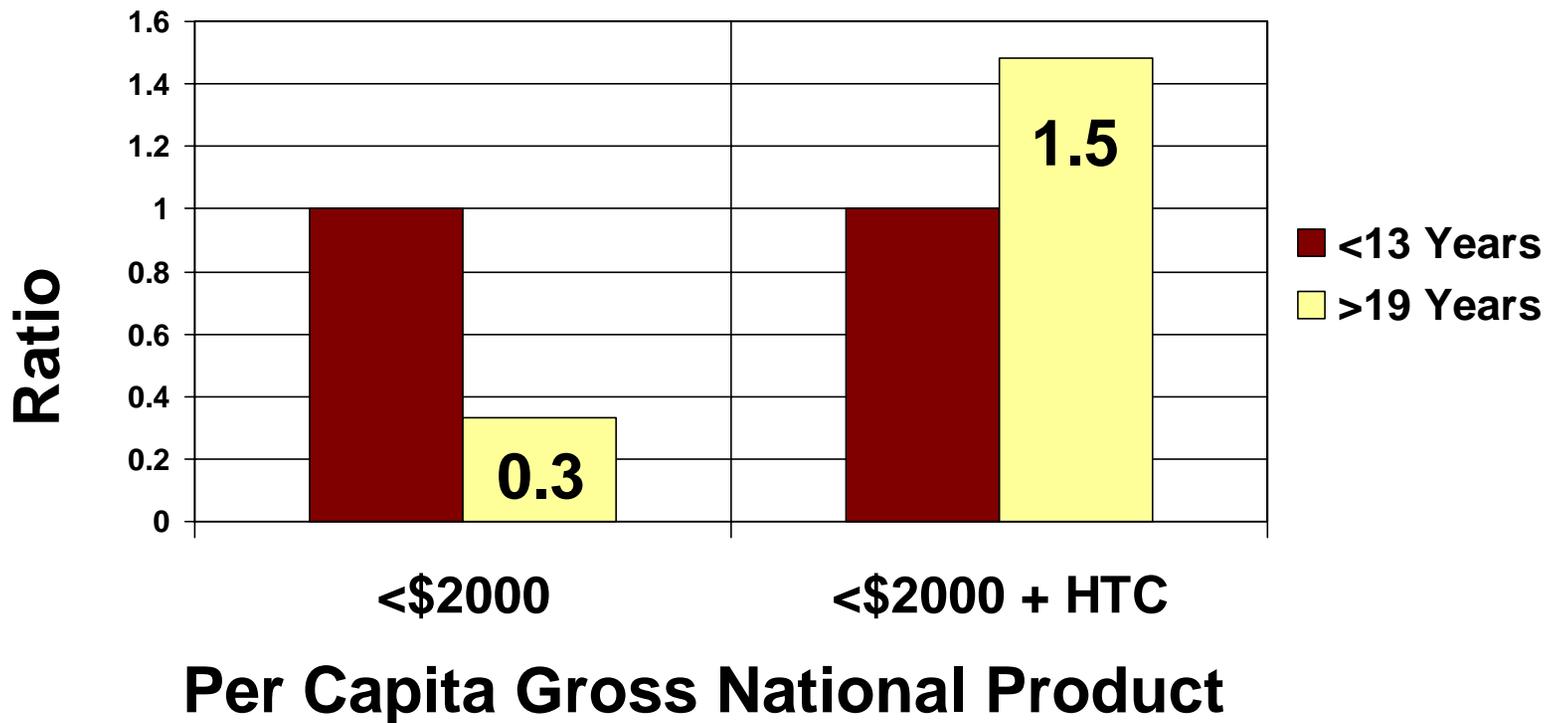
# Improving Survival into Adulthood

- **Government support**
- Hemophilia program within National health system
- Network of Treatment centres
- Provision of basic blood products
- Provision of concentrates
- Payment by Government/Social security/insurance



# RELATIONSHIP OF ECONOMIC CAPACITY AND NUMBER OF ADULT HEMOPHILIA PATIENTS:

## EFFECT OF HEMOPHILIA PROGRAMS





# Real Issue

Provision of safe, efficacious, affordable replacement therapy in adequate amounts as part of a sustainable national Hemophilia program.



# Improving Quality of Life

- **Government support**
- Network of treatment centres-decreases morbidity and mortality
- Access to concentrate for all
- Clinical monitoring of home therapy
- High users/surgery/inhibitors included
- UK-Treatment by postcode
- USA-Lifetime insurance ceilings
  - wide variation in distribution and reimbursement mechanisms



# Role of Government?

- National treatment strategy
- Ensure adequate provision of replacement therapy on a National basis
- Predict demand and supply required
- Expensive cases covered by central fund
- Plan for Emergencies
  
- National Tender/Central purchasing mechanism



# National Tenders

- Brings appropriate expertise together
- Improved selection criteria
- Improved assessment of products
- National assessment of demand and use
- Allows planning of cost and use
- Contingency planning
- Cost effective
- More uniform care nationally-optimises
- Allows manufacturers to predict demand and ensure supply



# National Tenders

- Limits clinical freedom ?
- Limits availability of different products ?
  
- Canada
- Ireland
- Brazil
- England



# Canada

- 10 Provinces-1 Tender
- Tender: -recombinant FVIII/FIX  
-Plasma derived FVIII/FIX  
-IVIG, Albumin  
-Toll Fractionation

Participants: CBS,Hemaquebec (6)  
Clinicians (2)  
Consumers (2)

3 Year Tender



# Canada

- Products licenced by Health Canada
- At least 2 products in each category where possible
- Clinical freedom maintained-90/95% Tender
- Contingency planning and distribution mechanisms greatly assisted with minimising impact of product shortages in 2001



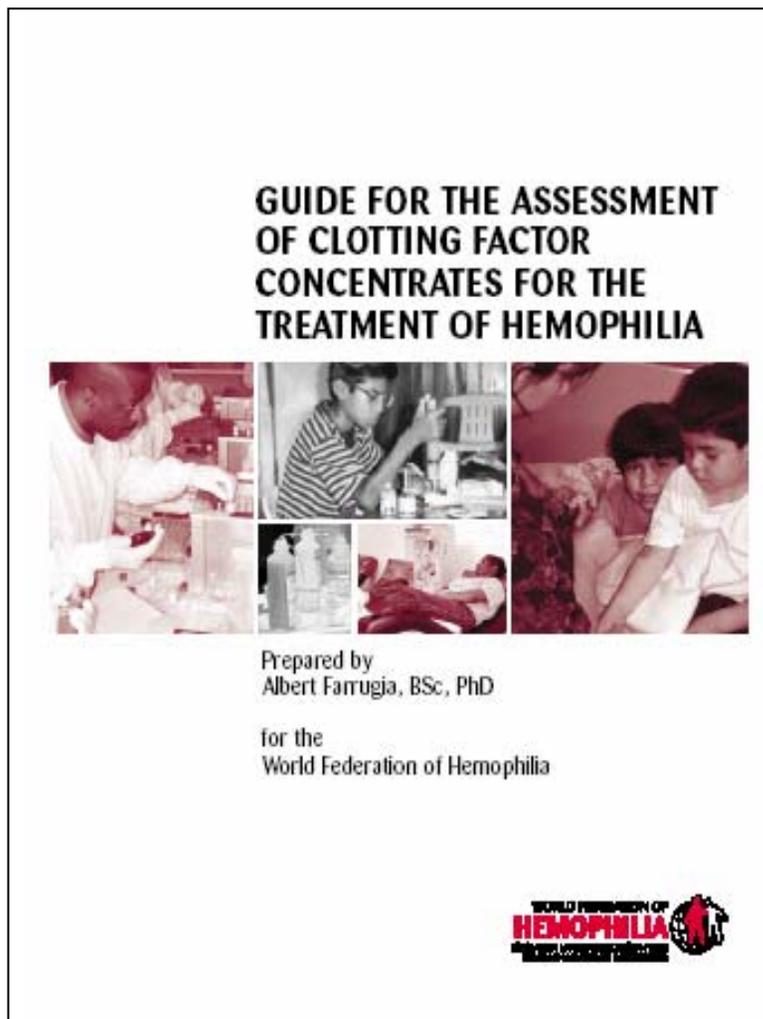
# Influence of Key Regulators

- FDA and EMEA play vital role Globally
- Decisions monitored by many countries
- Decisions impact products used in many countries
- Licencing confers reassurance



# WFH Regulatory Guidebook

Published to assist regulators to assess products not licenced by FDA or EMEA.





# Consultation

- **Decision criteria**
  - Safety,efficacy,supply,cost
  - evidence,data,science
  - Clinical reality
- **Poor Decision criteria**
  - Nationalism
  - Unrealistic timelines, aspirations
  - Emotion



# Global consultation-Objectives

- Consensus of understanding-if not policy
- Early warning system
- Optimise communication via regular contact
- Global ramifications of regulatory issues and decisions discussed and understood



# Global Consultation

## **PARTICIPANTS**

- WFH
- FDA
- EMEA
- WHO
- HHS
- DG SANCO
- PPTA
- EPFA
- IPOPI

## **REGIONAL**

- European network
- US network



# Global Consultation

## Standard Agenda

- Legislation-Directives,Guidelines,Regulations
  - EU ,USA ,Other
- Safety issues
- Availability issues
- Economic issues
- Global perspectives



# Thank You



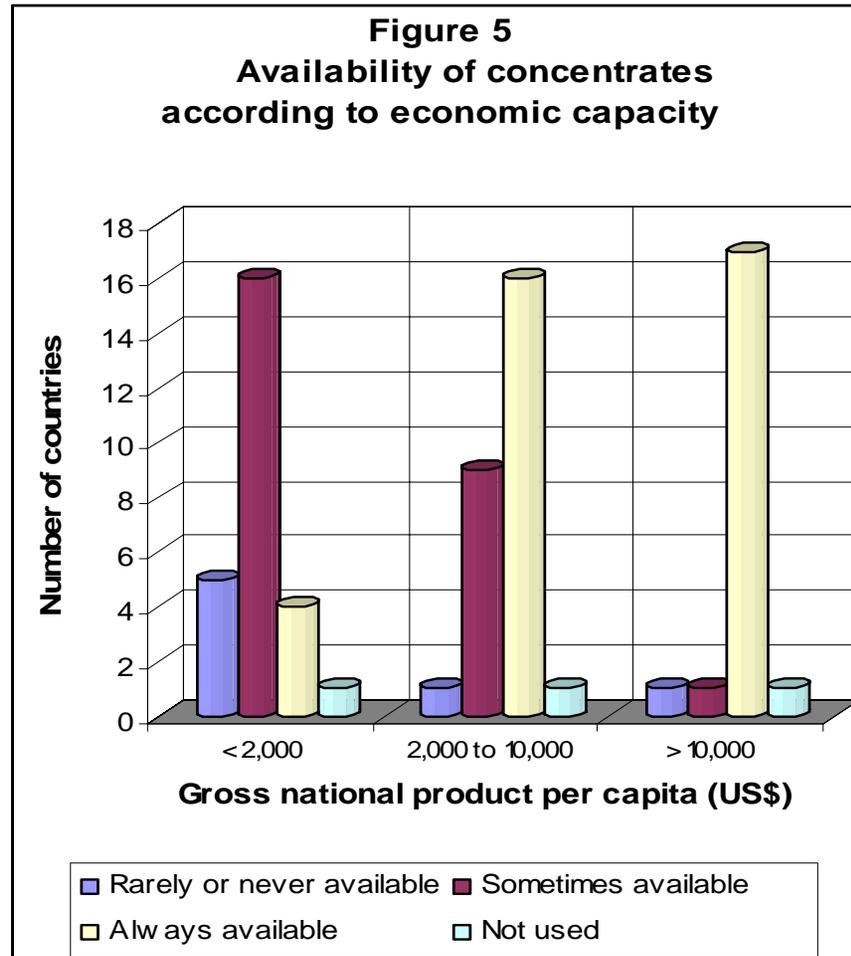
The logo consists of a stylized globe on the left, with two human figures in silhouette on the right. The figures are overlapping, with the one in front appearing to be holding the other's hand or arm. The entire graphic is rendered in a dark red color against a lighter red background.

WORLD FEDERATION OF  
**HEMOPHILIA**

FÉDÉRATION MONDIALE DE L'HÉMOPHILIE  
FEDERACION MUNDIAL DE HEMOFILIA

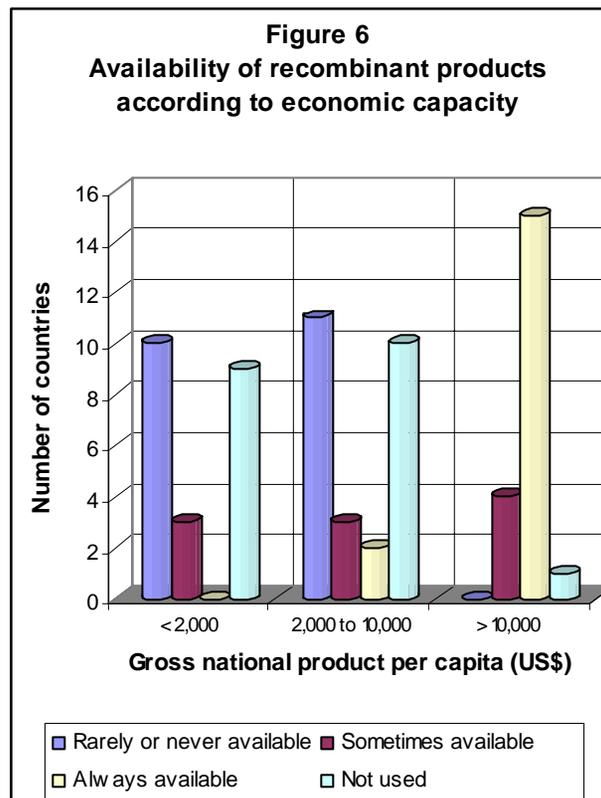


# Availability of factor Concentrates





# Availability of Recombinant Factor Concentrates





# Ireland

- Product selection and monitoring advisory group
- Set up under statutory instrument
- Recommended by Tribunal of inquiry
- Assisted with supply shortages in 2001/2002



# Ireland

## Participants

- Health officials (2)
- Regulatory authority (1)
- Clinicians (3)
- Hemophilia society (2)
- National transfusion service (2)
- Virologist (1)
- External experts (2)



# Ireland

## Tender

- Recombinant FVIII/FIX
- Plasma derived rarer factors
- Therapy for inhibitors
  
- EU procurement rules
- 2 year process



# Brazil/England

## Brazil

- Participants:
- Health ministry
- Clinicians
- Hemophilia society
- ?products licenced by FDA or EMEA

## England

- Tender for Recombinant-specific amount of money available, not for a specific amount of concentrate
- Age banding



# Consultation

- Timely, appropriate
- Involve consumers-lifetime users
- Involved in National tenders

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## **EU Blood Directive 2002/98/EC**

- Aspirations good, ignored reality
- Encourage voluntary unpaid donations
- Do not ban imports/limit supply
- Europe not self sufficient
- UK using US plasma due to vCJD risk